

## Accelerating Medicines Partnership: Type 2 Diabetes

The National Institutes of Health (NIH), 10 biopharmaceutical companies, and several non-profit organizations have designed an unprecedented new partnership. Managed through the Foundation for the NIH (FNIH), the Accelerating Medicines Partnership (AMP) brings high-level government, industry, and non-profit foundation partners together to identify and validate the most promising biological targets of disease for new diagnostic and drug development. The partners have designed a bold milestone-driven research plan to tackle this challenge for **type 2 diabetes**, as well as for Alzheimer's disease and the autoimmune disorders rheumatoid arthritis and systemic lupus erythematosus (lupus). What makes this public-private partnership unique is that AMP data will be considered precompetitive and made publicly accessible to the broad biomedical community for further research. This fact sheet addresses the AMP research plan for type 2 diabetes.

### Type 2 Diabetes

Type 2 diabetes (T2D) currently affects about 26 million people in the U.S. and more than 382 million people worldwide. As many as 79 million adults in the U.S. have "pre-diabetes" and are at high risk of developing T2D. Many complications are associated with the disease, including an increased risk for cardiovascular disease. Diabetes is a leading cause of kidney failure, lower limb amputations, and retinopathy, which can result in blindness.

### Need for New Therapies

While a number of therapies are approved for T2D, none can reverse disease progression over the long term or prevent complications of the disease. This deficiency is likely due to incomplete understanding of the many biological processes involved in disease development and progression. The research community has recently generated an enormous amount of genetic data on T2D in diverse populations that is unmatched in most other diseases. Much of the data is linked to clinical information, making it a rich resource for research aimed at understanding such a complex disease.

#### AMP Type 2 Diabetes: *The partners*

##### Government

- NIH

##### Industry

- Johnson & Johnson
- Lilly
- Merck
- Pfizer
- Sanofi

##### Non-Profit Organizations

- American Diabetes Association
- Foundation for the NIH

### AMP Approach

The approach of this project is to use and supplement the substantial amount of human genetic data available from people with T2D – or at risk for developing T2D – to identify and validate novel molecules and pathways as targets for therapeutic development. Genetic data paired with information on gene expression and epigenomics in relevant tissues, combined with clinical information, can provide clues about the function of genetic changes within an individual's genome that increases one's risk for developing T2D. After identifying DNA regions that might be critical for the development or progression of T2D, researchers will intensively analyze these DNA regions in targeted populations.

They will be looking for rare individuals whose genetic inheritance may provide a model of what targeted drug might achieve. In addition to leading to potential drug targets, this research will add to the foundational understanding of T2D.

#### *A T2D Knowledge Portal*

Researchers will build a database of DNA sequence, functional genomic and epigenomic information, and clinical data from studies on type 2 diabetes and its cardiac and renal complications, involving 100,000 – 150,000 individuals. The data set and analytical tools will be accessible to academic and industry researchers to identify and validate changes in DNA that spur the onset of diabetes, alter disease severity, speed or slow disease progression, or have a protective effect.

#### *Generating New Genomic Data*

As the Knowledge Portal is being mined and analyzed, the partnership will seek to fill in data gaps by executing specific analyses of the data. Specifically, researchers will investigate targets of particular interest by sequencing DNA from select individuals. For example, a DNA change that occurs in few individuals but has a significant effect on disease risk or progression can provide clues about the biological processes underlying the disease and uncover possible therapeutic targets.

### **Governance**

The steering committee (SC) for T2D will comprise representatives from NIH, FNIH, participating companies, and patient advocacy organizations. After AMP research grant awards are made, investigators carrying out the research will be added to the disease SCs. The SC is responsible for defining the research agenda and project plan, reviewing ongoing projects, and assessing milestones. The SC will operate under the direction of the overall AMP executive committee comprised of representatives from NIH, FNIH, participating companies, the Food and Drug Administration, and patient advocacy organizations.

### **Timeline and Deliverables**

This partnership will be a five-year initiative, beginning in mid-2014 with the selection of the institution to house the T2D knowledge portal. A parallel project to supplement existing data with new genomic data, including targets of interest, will begin in early 2015. Over the five years – through mid-2019 - the T2D SC will ensure substantial scientific and logistical interaction between the two projects.

### **Budget: 5 years (\$58.4 Million Total Project Funding)**

(\$Millions)	Total Project	Total NIH	Total Industry
<b>Type 2 Diabetes</b>	58.4	30.4	28